RFA 11-02 APPENDIX B: CIRM TRANSLATIONAL PORTFOLIO				
AWARD #	**PROGRAM**	**GOAL***	**DISEASE**	**APPROACH**
TR1-01273	Early Translation I	DC	Blood Disorder: Fanconi Anemia, XSCID	Autologous human iPSC-derived HSC genetically corrected by homologous recombination
DR1-01452	Disease Team	IND	Blood Disorder: Sickle Cell Disease	Autologous human HSC, genetically corrected ex vivo by lentiviral vector mediated addition of a hemoglobin gene that blocks sickling. IV administration after myeloablation
TR1-01216	Early Translation I	DC	Bone/Cartilage Disorders: Focal cartilage defect, osteoarthritis	Human iPSC- or ESC derived chrondrocyte progenitors implanted into chrondral defect or injected into OA joint
TR2-01829	Early Translation II	DC	Bone/Cartilage Disorders: Osteoarthritis	Optimized small molecule of lead molecule PRO1 that induces chrondrocyte differentiation of resident human MSC
TR2-01780	Early Translation II	DCF	Bone/Cartilage Disorders: Osteoporosis-related vertebral compression fractures	Human MSC in combination with PTH (parathyroid hormone)
TR2-01821	Early Translation II	DC	Bone/Cartilage Disorders: Spinal fusion	Autologous human adult perivascular stem cells and an osteoinductive protein on a FDA-approved acellular scaffold
TR2-01816	Early Translation II	DC	Cancer: Hematologic malignancy (AML, ALL)	Small molecule inhibitor of BCL6 targeting human CSC
DR1-01430	Disease Team	IND	Cancer: Hematologic malignancy (AML, CML, ALL, CLL)	Existing candidate molecules (3 small molecule, 3 MAb) targeting leukemic stem cells (LSC) by blocking survival and self-renewal pathways that function preferentially in human CSC compared to normal HSC
DR1-01485	Disease Team	IND	Cancer: Hematologic malignancy (AML)	Monoclonal antibody against human CD47 – "Don't eat me" antigen that is expressed on LSC and inhibits their phagocytosis by macrophages
TR2-01789	Early Translation II	DC	Cancer: Hematologic malignancy (CML)	Small molecule pan BCL-2 inhibitor targeting human CSC
DR1-01477	Disease Team	IND	Cancer: Solid tumor (colon, ovarian)	Small molecules specific for either of two drug targets in CSC
DR1-01421	Disease Team	IND	Cancer: Solid tumor (Glioblastoma)	Allogeneic established hNSC line to target tumor, engineered ex vivo to deliver carboxylesterase to locally convert CPT-11 to more potent SN-38
DR1-01426	Disease Team	IND	Cancer: Solid tumor (Glioblastoma)	Best of allogeneic hNSC, adult or fetal, or hMSC to target tumor, engineered ex vivo to deliver a tumorcidal gene product, TRAIL or cytosine deaminase, and a suicide gene delivered either intratumoral or intravscular

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TR2-01791	Early Translation II	DC	Cancer: Solid tumor (Glioblastoma)	Tumor homing by hMSC genetically engineered to produce replication competent retrovirus encoding a suicide gene
DR1-01423	Disease Team	IND	Diabetes: Type 1	Allogeneic hESC-derived pancreatic cell progenitors in a device implanted sc that mature in vivo to beta cells that secrete insulin in response to glucose. Transient immunosuppression
DR1-01444	Disease Team	IND	Eye Disease: Age-related macular degeneration (dry form)	Allogeneic functionally polarized hESC-derived RPE monolayers on synthetic substrate implanted sub-retinally
TR1-01219	Early Translation I	DC	Eye Disease: Age-related macular degeneration (dry form)	Autologous human iPSC-derived RPE (generated without integrating vectors)
TR1-01272	Early Translation I	DC	Eye Disease: Age-related macular degeneration (dry form)	Autologous human adult SC (CMZ) or iPSC-derived RPE +/- ex vivo engineering to express negative regulators of complement cascade
TR2-01768	Early Translation II	DCF	Eye Disease: Limbal stem cell deficiency	Ex vivo expansion of human corneal epithelial stem/progenitor cells, also known as limbal stem cells (LSC)
TR2-01794	Early Translation II	DC	Eye Disease: Retinitis Pigmentosa	Allogenic human retinal progenitor cells
DR1-01461	Disease Team	IND	Heart Disease: Advanced ischemic cardiomyopathy	Autologous human cardiac derived cells, 'cardiospheres', expanded and delivered by direct catheter injection into heart muscle
DR1-01431	Disease Team	IND	HIV/AIDS	Autologous human HSC transduced ex vivo with a lentiviral vector engineered to express an shRNA against CCR5 & a fusion inhibitor. IV administration after myeloablation
DR1-01490	Disease Team	IND	HIV/AIDS	Autologous human HSC transduced ex vivo with non-integrating vector engineered to express a zinc finger nuclease targeting CCR5. IV administration after myeloablation
TR2-01771	Early Translation II	DC	HIV/AIDS	Autologous human HSC genetically modified with multiple anti-HIV resistance genes and a drug resistance gene
TR2-01857	Early Translation II	DC	Liver Failure	Allogeneic genetically modified hESC-derived hepatocytes
TR1-01249	Early Translation I	DC	Multiple	Recombinant Wnt in a sustained release formulation to stimulate endogenous stem cells to repair tissue
TR2-01844	Early Translation II	DC	Neurodegenerative Disease - Spinal Muscular Atrophy	Small molecule that increases SMN1 in patient iPSC-derived motor neurons

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DR1-01471	Disease Team	IND	Neurodegenerative Disease: ALS	Allogeneic hESC-derived astrocyte precursors delivered into spinal cord (delivery device)
TR1-01245	Early Translation I	DC	Neurodegenerative Disease: Alzheimer's Disease	Allogeneic hESC-derived NSC
TR2-01832	Early Translation II	DCF	Neurodegenerative Disease: Canavan Disease	Autologous iPSC-derived neural or oligodendrocyte progenitors, genetically modifed to correct mutant aspartoacylase (ASPA) gene
TR1-01257	Early Translation I	DC	Neurodegenerative Disease: Huntington's Disease	Allogeneic hMSC engineered ex vivo to express siRNA targeting mutant huntingtin mRNA. Injected intracranially
TR2-01841	Early Translation II	DC	Neurodegenerative Disease: Huntington's Disease	allogeneic hESC-derived neural stem or progenitor cells for transplantation
TR1-01267	Early Translation I	DC	Neurodegenerative Disease: Parkinson's Disease	hNSC derived from best of adult SC, hESC, iPSC
TR2-01778	Early Translation II	DCF	Neurodegenerative Disease: Parkinson's Disease	Small molecule modulator of neuroinflammation identified by screening on astrocytes/microglial from patient derived iPSC
TR2-01856	Early Translation II	DC	Neurodegenerative Disease: Parkinson's Disease	Allogeneic hESC-derived dopaminergic neurons
TR2-01749	Early Translation II	DCF	Neurological Disorder: Refractory epilepsy	hESC-derived progenitors of GABAergic inhibitory neurons analogous to those isolated from medial ganglionic eminence
TR2-01767	Early Translation II	DCF	Neurological Disorder: Traumatic brain injury	Allogeneic hESC-derived NSC
TR2-01814	Early Translation II	DCF	Neurological Disorders: Autism Spectrum Disorders	Neurons from ASD (and control) iPSC for phenotype screening, assay development and validation, drug screening and biomarker identification
CT1-05168	Targeted Clinical Development	complete Ph1 study in 3 cohorts	Neurological Disorders: subacute SCI (complete thoracic injury, complete cervical injury, incomplete thoracic injury)	h-ESC-derived oligodendrocyte progenitor cells
TR2-01785	Early Translation II	DCF	Neurological Disorders: SCI (conus medullaris/cauda equina, CM/CE, injury)	hESC-derived motor and autonomic precursor neurons
TR2-01756	Early Translation II	DCF	Skeletal muscle disorders: Duchenne muscular dystrophy	Autologous skeletal muscle precursor cells derived from human iPSC genetically modifed to correct the dystrophin gene

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DR1-01454	Disease Team	IND	Skin Disease: Epidermolysis bullosa	Epidermal sheets from expanded autologous genetically corrected (to express wt COL7A1) iPSC-derived keratinocytes
TR2-01787	Early Translation II	DC	Diabetes: Wound healing, Chronic Diabetic foot ulcers	Allogenic hMSC on a dermal regeneration scaffold
DR1-01480	Disease Team	IND	Neurolgical Disorders: Stroke	Allogeneic hESC-derived NSC line alone or in combination with matrix

* The Project Goal is:
IND - file an approvable IND with the FDA;
DC - achieve a development candidate ready for IND-enabling preclinical development
DCF - show feasibility of a potential development candidate by achieving initial proof of concept